



Application Serial No.: 09/155,708
Amendment and Response dated March 4, 2004
Reply to Office Action of December 4, 2003
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Amendments To The Claims:

This listing of claims will replace all prior versions, and listings, of claims in the application:

Listing of Claims:

Claims 1-11 (Canceled)

Claim 12 (currently amended): A method for preparing a suppression effector and replacement nucleic acid, said method comprising:

a) preparing a suppression effector that binds to a coding region of a mature RNA encoding a mutant allele, thereby to inhibit the expression of the mutant allele, wherein the suppression effector is a nucleic acid or a peptide nucleic acid; and

b) preparing a replacement nucleic acid that encodes a wild-type or non-disease causing allele and that comprises at least one degenerate / wobble nucleotide that is altered so that the replacement nucleic acid is not suppressed, or is only partially suppressed, by the suppression effector.

Claim 13 (currently amended): A method for preparing a suppression effector and replacement nucleic acid, the method comprising:

a) preparing a ribozyme that cleaves a mature RNA encoding a mutant allele; and

b) preparing a replacement nucleic acid that encodes a wild-type or non-disease causing allele and that comprises at least one degenerate / wobble nucleotide that is altered so that the replacement nucleic acid is not suppressed, or is only partially suppressed, by the ribozyme.

Claim 14 (Canceled)

Claim 15 (Canceled)

Claim 16 (previously presented): The method of claim 12, wherein the suppression effector is a nucleic acid that forms a triple helix with the mutant allele.

Claim 17 (previously presented): The method of claim 12, wherein the suppression effector is an nucleic acid.

Claim 18 (previously presented): The method of claim 12, wherein the suppression effector is a single-stranded RNA.

Claim 19 (previously presented): The method of claim 12, wherein the suppression effector is a ribozyme that cleaves an RNA encoded by the mutant allele.

Claim 20 (previously presented): The method of claim 19, wherein the ribozyme cleaves the RNA at an NUX ribozyme cleavage site.

Claim 21 (previously presented): The method of claim 12 or 13, wherein the suppression effector is operatively linked to an expression vector.

Claim 22 (Canceled)

Claim 23 (Canceled)

Claim 24 (Canceled)

Claim 25 (currently amended): The method of claim 12 or 13, wherein the replacement nucleic acid encodes a protein selected from the group consisting of mammalian rhodopsin, collagen 1A1, collagen 1A2, and peripherin.

Claim 26 (previously presented): The method of claim 12 or 13, wherein the replacement nucleic acid is operatively linked to an expression vector.

Claim 27 (previously presented): The method of claim 21, wherein the expression vector is a viral expression vector.

Claim 28 (Canceled)

Claim 29 (Canceled)

Claim 30 (Canceled)

Claim 31 (Canceled)

Claim 32 (previously presented): The kit of claim 44, wherein the suppression effector is a nucleic acid that forms a triple helix with the mutant allele.

Claim 33 (previously presented): The kit of claim 44, wherein the suppression effector is an nucleic acid.

Claim 34 (previously presented): The kit of claim 44, wherein the suppression effector is a single-stranded RNA.

Claim 35 (previously presented): The kit of claim 44, wherein the suppression effector is a ribozyme that cleaves an RNA encoded by the mutant allele.

Claim 36 (previously presented): The kit of claim 35, wherein the ribozyme cleaves the RNA at an NUX ribozyme cleavage site.

Claim 37 (previously presented): The kit of claim 44, wherein the suppression effector is operatively linked to an expression vector.

Claim 38 (Canceled)

Claim 39 (Canceled)

Claim 40 (Canceled)

Claim 41 (currently amended): The kit of claim 44 or 45, wherein the replacement nucleic acid encodes a protein selected from the group consisting of mammalian rhodopsin, collagen 1A1, collagen 1A2, and peripherin.

Claim 42 (previously presented): The kit of claim 44 or 45, wherein the replacement nucleic acid is operatively linked to an expression vector.

Claim 43 (previously presented): The kit of claim 42, wherein the expression vector is a viral expression vector.

Claim 44 (currently amended): A kit comprising:

a suppression effector that ~~suppresses the expression~~ binds to the coding region of a mature RNA encoding a mutant allele, wherein the suppression effector is a nucleic acid or a peptide nucleic acid; and

a replacement nucleic acid that encodes a wild-type or non-disease causing allele that is not suppressed, or is only partially suppressed, by the suppression effector and that differs from the mutant allele in at least one degenerate / wobble nucleotide.

Claim 45 (previously presented): A kit comprising:

at least one ribozyme that cleaves a mature RNA encoding a mutant allele; and

a replacement nucleic acid that encodes a wild-type or non-disease causing allele and that is not suppressed, or is only partially suppressed, by the suppression effectors, wherein the replacement nucleic acid differs from the mutant allele in at least one degenerate / wobble nucleotide.

Claim 46 (currently amended): A ribozyme comprising a nucleotide sequence encoded by nucleotides 101 - 137 of SEQ ID NO:4.

Claim 47 (currently amended):. A ribozyme comprising a nucleotide sequence encoded by nucleotides 116 - 153 of SEQ ID NO:14.

Claim 48 (currently amended): A ribozyme comprising a nucleotide sequence encoded by nucleotides 112 - 148 of SEQ ID NO:15.

Claim 49 (currently amended): A ribozyme comprising a nucleotide sequence encoded by nucleotides 107 - 141 of SEQ ID NO:18.

Claim 50 (previously presented): The method of claim 26, wherein the expression vector is a viral expression vector.

Claim 51 (currently amended): A composition, the composition comprising:

a) a suppression effector that binds to the coding region of a mature RNA encoding a mutant allele, thereby to inhibit the expression of the mutant allele, wherein the suppression effector is a nucleic acid or a peptide nucleic acid; and

b) a replacement nucleic acid that encodes a wild-type or non-disease causing allele and that comprises at least one degenerate / wobble nucleotide that is altered so that the replacement nucleic acid is not suppressed, or is only partially suppressed, by the suppression effector.

Claim 52 (previously presented): A composition, the composition comprising:

a) a ribozyme that cleaves a mature RNA encoding a mutant allele; and

b) a replacement nucleic acid that encodes a wild-type or non-disease causing allele and that comprises at least one degenerate / wobble nucleotide that is altered so that the replacement nucleic acid is not suppressed, or is only partially suppressed, by the ribozyme.

Claim 53 (Canceled)

Claim 54 (previously presented): The composition of claim 51, wherein the suppression effector is a nucleic acid that forms a triple helix with the mutant allele.

Claim 55 (currently amended): The composition of claim 51, wherein the suppression effector is an a nucleic acid.

Claim 56 (previously presented): The composition of claim 51, wherein the suppression effector is a single-stranded RNA.

Claim 57 (previously presented): The composition of claim 51, wherein the suppression effector is a ribozyme that cleaves an RNA encoded by the mutant allele.

Claim 58 (previously presented): The composition of claim 57, wherein the ribozyme cleaves the RNA at an NUX ribozyme cleavage site.

Claim 59 (previously presented): The composition of claim 51, wherein the suppression effector is operatively linked to an expression vector.

Claim 60 (previously presented): The composition of claim 52, wherein the ribozyme is operatively linked to an expression vector.

Claim 61 (previously presented): The composition of claim 59 or 60, wherein the expression vector is a viral expression vector.

Claim 62 (currently amended): The composition of claim 51 or 52, wherein the replacement nucleic acid encodes a protein selected from the group consisting of mammalian rhodopsin, collagen 1A1, collagen 1A2, and peripherin.

Claim 63 (previously presented): The composition of claim 51 or 52, wherein the replacement nucleic acid is operatively linked to an expression vector.

Claim 64 (previously presented): The composition of claim 63, wherein the expression vector is a viral expression vector.

Claim 65 (currently amended): The composition of claim 52, wherein the ribozyme comprises a nucleotide sequence encoded by a sequence selected from the group consisting of nucleotides 101 - 137 of SEQ ID NO:4, nucleotides 116 - 153 of SEQ ID NO:14, nucleotides 112 - 148 of SEQ ID NO:15, and nucleotides 107 - 141 of SEQ ID NO:18.

Claim 66 (currently amended): The method of claim 13, wherein the ribozyme comprises a nucleotide sequence encoded by a sequence selected from the group consisting of nucleotides 101 - 137 of SEQ ID NO:4, nucleotides 116 - 153 of SEQ ID NO:14, nucleotides 112 - 148 of SEQ ID NO:15, and nucleotides 107 - 141 of SEQ ID NO:18.

Claim 67 (currently amended): The kit of claim 45, wherein the ribozyme comprises a nucleotide sequence encoded by a sequence selected from the group consisting of nucleotides 101 - 137 of SEQ ID NO:4, nucleotides 116 - 153 of SEQ ID NO:14, nucleotides 112 - 148 of SEQ ID NO:15, and nucleotides 107 - 141 of SEQ ID NO:18.

Claim 68 (previously presented): The kit of claim 45, wherein the ribozyme is operatively linked to an expression vector.

Claim 69 (previously presented): The kit of claim 37 or 68, wherein the expression vector is a viral expression vector.

Claim 70 (previously presented): The method of claim 12 or 13, wherein the suppression effector suppresses both alleles of an endogenous gene.

Claim 71 (previously presented): The kit of claim 44, wherein the suppression effector suppresses both alleles of an endogenous gene.

Claim 72 (previously presented): The kit of claim 45, wherein the ribozyme suppresses both alleles of an endogenous gene.

Claim 73 (previously presented): The composition of claim 51, wherein the suppression effector suppresses both alleles of an endogenous gene.

Claim 74 (previously presented): The composition of claim 52, wherein the ribozyme suppresses both alleles of an endogenous gene.

Claim 75 (previously presented): The method of claim 12 or 13, wherein the RNA is an mRNA.

Claim 76 (previously presented): The kit of claim 44 or 45, wherein the RNA is an mRNA.

Claim 77 (previously presented): The composition of claim 51 or 52, wherein the RNA is an mRNA.